

CIRM Board Invests \$7 Million in Great New Ideas and Treatment for Deadly Brain Cancer

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Oakland, CA – Some of the biggest advances in science began with a simple idea that built on previous knowledge or that challenged current thinking by setting out in a completely different direction. Today the governing Board of the California Institute for Regenerative Medicine (CIRM), approved more than \$4 million in funding for 19 projects that offer new ideas and approaches to issues as varied as stroke, heart disease, prostate cancer and blindness.

The awards are part of CIRM's Discovery Inception program. This provides seed funding for great ideas that could impact stem cell research but need modest support to test those ideas. CIRM's support will help researchers gather the data necessary to enable them to apply for larger funding opportunities, from CIRM and other institutions, in the future.

"This is a program supporting early stage ideas that have the potential to be ground breaking," says C. Randal Mills, Ph.D., President and CEO of CIRM. "We asked scientists to pitch us their best new ideas, things they want to test but that are hard to get funding for. We know not all of these will pan out, but those that do succeed have the potential to advance our understanding of stem cells and hopefully lead to treatments in the future."

Among the successful applications is Alysson Muotri at UC San Diego. He has identified some anti-retroviral drugs – already approved by the Food and Drug Administration (FDA) – that could help stop inflammation in the brain. This kind of inflammation is an important component in several diseases such as Alzheimer's, autism, Parkinson's, Lupus and Multiple Sclerosis. Alysson wants to find out why and how these drugs helps reduce inflammation and how it works. If he is successful it is possible that patients suffering from brain inflammation could immediately benefit from some already available anti-retroviral drugs.

Stanley Carmichael at UC Los Angeles wants to use induced pluripotent stem (iPS) cells – these are adult cells that have been genetically re-programmed so they are capable of becoming any cell in the body – to see if they can help repair the damage caused by a stroke. With stroke the leading cause of adult disability in the US there is clearly a big need for this kind of big idea.

Holger Willenbring at UC San Francisco wants to use stem cells to create a kind of mini liver, one that can help patients whose own liver is being destroyed by disease. The mini livers could, theoretically, help stabilize a person's own liver function until a transplant donor becomes available or even help them avoid the need for liver transplantation in the first place. Considering that every year, one in five patients on the US transplant waiting list will die or become too sick for transplantation, this kind of research could have enormous life-saving implications.

The CIRM Board also approved \$2.9 million for an immune-system boosting vaccine that targets cancer stem cells in glioblastoma, the most common and the deadliest form of brain cancer. This came under CIRM's Translation program which helps promising stem cell-based projects complete the research necessary to advance into a clinical trial. The research will be done by Albert Wong, M.D, a neurosurgeon at Stanford University.

"Glioblastoma is a particularly aggressive form of brain tumor," says Mills. "Average life expectancy for those diagnosed with this cancer is less than 18 months so clearly there's a real need for new, more effective therapies."

The successful applications under the Discovery Inception program are:

Application	Researcher	Institution	ICOC Committed funding

DISC1-08825	Alysson Muotri	U.C. San Diego	\$232,200
08723	Stanley Carmichael	U.C. Los Angeles	\$229,396
08792	Holger Willenbring	U.C. San Francisco	\$206,460
08731	Bertha Chen	Stanford University	\$237,564
08848	Jeffrey Goldberg	Stanford University	\$237,564
08800	Clive Svendsen	Cedars-Sinai	\$241,992
08855	Marcus Muench	Blood Systems Research Institute	\$180,000
08652	Andrew Brack	U.C. San Francisco	\$180,000
08819	Bennet Novitch	U.C. Los Angeles	\$230,400
08790	Arjun Deb	U.C. Los Angeles	\$230,400
08842	Owen Witte	U.C. Los Angeles	\$230,400
08650	Phillip Yang	Stanford University	\$236,338
08823	April Pyle	U.C. Los Angeles	\$230,400
08868	Senta Georgia	Children's Hospital of Los Angeles	\$180,000
08737	Dionicio Siegel	U.C. San Diego	\$232,200
08683	Karl Wahlin	U.C. San Diego	\$232,200
08750	Philip Beachy	Stanford University	\$237,564
08776	Jacob Corn	U.C. Berkeley	\$235,800
08643	Linda Cambier	Cedars-Sinai Medical Center	\$181,063

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs, and act with a sense of urgency to succeed in that mission.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today's most promising stem cell technologies.

With \$3 billion in funding and approximately 300 active stem cell programs in our portfolio, CIRM is the world's largest institution dedicated to helping people by bringing the future of cellular medicine closer to reality.

For more information, go to www.cirm.ca.gov.

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